This information was supplied by the NC Medicaid Division of Health Benefits.

NC Division of Medical Assistance **Outpatient Pharmacy** **Prior Approval Criteria** **Exondys 51**	**Medicaid and Health Choice** **Effective Date: May 1, 2017**

Therapeutic Class Code: Z1R
Therapeutic Class Description: GENETIC D/O TX-EXON SKIPPING ANTISENSE OLIGONUCLEOTIDE

Medication	Generic Code Number(s)	NDC Number(s)
Exondys 51	42296, 42295	

Eligible Beneficiaries
NC Medicaid (Medicaid) beneficiaries shall be enrolled on the date of service and may have service restrictions due to their eligibility category that would make them ineligible for this service.

NC Health Choice (NCHC) beneficiaries, ages 6 through 18 years of age, shall be enrolled on the date of service to be eligible, and must meet policy coverage criteria, unless otherwise specified. **EPSDT does not apply to NCHC beneficiaries**.

EPSDT Special Provision: Exception to Policy Limitations for Beneficiaries under 21 Years of Age 42 U.S.C. § 1396d(r) [1905(r) of the Social Security Act]

Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) is a federal Medicaid requirement that requires the state Medicaid agency to cover services, products, or procedures for Medicaid beneficiaries under 21 years of age **if** the service is **medically necessary health care** to correct or ameliorate a defect, physical or mental illness, or a condition [health problem] identified through a screening examination (includes any evaluation by a physician or other licensed clinician). This means EPSDT covers most of the medical or remedial care a child needs to improve or maintain his/her health in the best condition possible, compensate for a health problem, prevent it from worsening, or prevent the development of additional health problems. Medically necessary services will be provided in the most economic mode, as long as the treatment made available is similarly efficacious to the service requested by the beneficiary's physician, therapist, or other licensed practitioner; the determination process does not delay the delivery of the needed service; and the determination does not limit the beneficiary's right to a

free choice of providers.
EPSDT does not require the state Medicaid agency to provide any service, product, or procedure
a. that is unsafe, ineffective, or experimental/investigational.
b. that is not medical in nature or not generally recognized as an accepted method of medical practice or treatment.

Service limitations on scope, amount, duration, frequency, location of service, and/or other specific criteria described in clinical coverage policies may be exceeded or may not apply as long as the provider's documentation shows that the requested service is medically necessary "to correct or ameliorate a defect, physical or mental illness, or a condition" [health problem]; that is, provider documentation shows how the service, product, or procedure meets all EPSDT criteria, including to correct or improve or maintain the beneficiary's health in the best condition possible, compensate fora health problem, prevent it from worsening, or prevent the development of additional health problems.

EPSDT and Prior Approval Requirements

EPSDT DOES NOT ELIMINATE THE REQUIREMENT FOR PRIOR APPROVAL IF PRIOR APPROVAL IS REQUIRED. Additional information on EPSDT guidelines may be accessed at https://medicaid.ncdhhs.gov/medicaid/get-started/find-programs-and-services-right-you/medicaid-benefit-children-and-adolescents.

Criteria for Initial Coverage:

- The beneficiary has a diagnosis of Duchenne Muscular Dystrophy
- Medical records are submitted (ex chart notes, laboratory values) that confirm the mutation of the Duchenne Muscular Dystrophy gene is amenable to exon 51 skipping
- Medication is prescribed by or in consultation with a neurologist
- The beneficiary is not taking Exondys 51 with any other RNA antisense agent, or any other gene therapy
- Exondys 51 dosing for Duchenne Muscular Dystrophy is in accordance with the USFDA approved labeling: maximum dosing of 30mg/kg once weekly
- Maximum length of initial approval: 6 months

Criteria for Renewal Coverage:

- Documentation must be submitted that shows the beneficiary:
  - Has shown an improvement in dystrophin levels **OR**
  - Is not ventilator dependent **OR**
  - Has some functional use of upper extremities **OR**
  - Has an ability to walk with or without assistive devices

References

1. Prescriber Information Exondys 51 ® (eteplirsen) Sarepta Therapeutics, Inc, Cambridge, MA 02142. September 2016.

Criteria Change Log

05/01/2017	Criteria effective date